

Union Calendar No. 300

100TH CONGRESS
1ST SESSION**H. R. 3459****[Report No. 100-473]**

To amend the Federal Food, Drug, and Cosmetic Act to revise the provisions respecting orphan drugs and for other purposes.

IN THE HOUSE OF REPRESENTATIVES

OCTOBER 9, 1987

Mr. WAXMAN introduced the following bill; which was referred to the Committee on Energy and Commerce

DECEMBER 10, 1987

Reported with an amendment, committed to the Committee of the Whole House on the State of the Union, and ordered to be printed

[Strike out all after the enacting clause and insert the part printed in italic]

[For text of introduced bill, see copy of bill as introduced on October 9, 1987]

A BILL

To amend the Federal Food, Drug, and Cosmetic Act to revise the provisions respecting orphan drugs and for other purposes.

1 *Be it enacted by the Senate and House of Representa-*
2 *tives of the United States of America in Congress assembled,*

3 **SECTION 1. SHORT TITLE.**

4 *This Act may be cited as the "Orphan Drug Amend-*
5 *ments of 1987".*

1 **SEC. 2. MARKET PROTECTION.**

2 (a) *LIMIT.*—

3 (1) *Section 527(a) of the Federal Food, Drug,*
4 *and Cosmetic Act (21 U.S.C. 360cc(a)) is amended to*
5 *read as follows:*

6 “(a) *Except as provided in subsection (b), if the*
7 *Secretary—*

8 “(1) *approves an application filed under section*
9 *505(b) for a drug designated under section 526 for a*
10 *rare disease or condition and the application is the*
11 *first section 505(b) application approved for such drug*
12 *for such disease or condition, then during the 7-year*
13 *period beginning on the date the Secretary approved*
14 *such first application—*

15 “(A) *the Secretary may approve for any*
16 *person an application under section 505(b) (other*
17 *than an application which includes a certification*
18 *described in section 505(b)(2)) for such drug for*
19 *such disease or condition, and*

20 “(B) *the Secretary may not approve—*

21 “(i) *an application under section*
22 *505(j), or*

23 “(ii) *an application which includes a*
24 *certification described in section 505(b)(2)*
25 *for such drug for such disease or condition, for a*
26 *person who is not the holder of an approved appli-*

1 *cation under section 505(b) for such drug for such*
2 *disease or condition,*

3 *“(2) issues a certification under section 507 for a*
4 *drug designated under section 526 for a rare disease or*
5 *condition and the certification is the first certification*
6 *issued under section 507 for such drug for such disease*
7 *or condition, then during the 7-year period beginning*
8 *on the date the Secretary issued such first*
9 *certification—*

10 *“(A) the Secretary may approve for any*
11 *person an application (other than an application*
12 *described in subparagraph (B)) for a certification*
13 *of such drug for such disease or condition under*
14 *section 507, and*

15 *“(B) the Secretary may not issue a certifica-*
16 *tion with respect to—*

17 *“(i) an abbreviated application filed*
18 *under section 507, or*

19 *“(ii) an application filed under section*
20 *507 which includes investigations of the type*
21 *described in section 505(b)(2)*

22 *for such drug for such disease or condition for a*
23 *person who is not the holder of an approved certi-*
24 *fication under section 507 for such drug for such*
25 *disease or condition, or*

1 “(3) issues a license under section 351 of the
2 *Public Health Service Act* for a drug designated under
3 section 526 for a rare disease or condition and the li-
4 cense is the first license issued under such section 351
5 for such drug for such disease or condition, then
6 during the 7-year period beginning on the date the
7 Secretary issued such first license—

8 “(A) the Secretary may approve for any
9 person an application (other than an application
10 described in subparagraph (B)) for a license
11 under such section 351 for such drug for such dis-
12 ease or condition, and

13 “(B) the Secretary may not approve an ap-
14 plication for a license for such drug for such dis-
15 ease or condition which is of the type which in-
16 cludes investigations of the type described in sec-
17 tion 505(b)(2) for a person who is not the holder
18 of a license issued under such section 351 for
19 such drug for such disease or condition.”.

20 (2) Section 527(b) of such Act (21 U.S.C.
21 360cc(b)) is amended—

22 (A) by striking out “505” the first place it
23 appears and inserting in lieu thereof “505(b)”,
24 and

1 (B) by inserting after “issuance of the li-
 2 cense” the following: “and notwithstanding sub-
 3 section (a)”.

4 (b) *TECHNICALS*.—

5 (1) Section 525(a) of the Federal Food, Drug,
 6 and Cosmetic Act (21 U.S.C. 360aa(a)) is amended
 7 by striking out “505” each place it appears and insert-
 8 ing in lieu thereof “505(b)”.

9 (2) Section 526(a)(1)(A) of the Federal Food,
 10 Drug, and Cosmetic Act (21 U.S.C. 360bb(a)(1)(A))
 11 is amended by striking out “505” and inserting in lieu
 12 thereof “505(b)”.

13 (3) Section 527 of the Federal Food, Drug, and
 14 Cosmetic Act (21 U.S.C. 360cc) is amended—

15 (A) by striking out the second comma after
 16 “507” the third time it appears in subsection (b),
 17 and

18 (B) by striking out “(1) The” in subsection
 19 (b)(1) and inserting in lieu thereof “(1) the”.

20 **SEC. 3. DESIGNATION AS AN ORPHAN DRUG.**

21 (a) *REQUEST*.—Section 526(a)(1) of the Federal Food,
 22 Drug, and Cosmetic Act (21 U.S.C. 360bb(a)(1)) is amend-
 23 ed by adding after the first sentence the following: “A request
 24 for designation of a drug shall be made before the submission
 25 of an application under section 505(b) for the drug, the sub-

1 mission of an application for certification of the drug under
2 section 507, or the submission of an application for licensing
3 of the drug under section 351 of the Public Health Service
4 Act.”.

5 (b) *DISCONTINUANCE*.—Section 526 of the Federal
6 Food, Drug, and Cosmetic Act (21 U.S.C. 360bb) is amend-
7 ed by redesignating subsections (b) and (c) as subsections (c)
8 and (d), respectively, and by adding after subsection (a) the
9 following:

10 “(b) A designation of a drug under subsection (a) shall
11 be subject to the condition that—

12 “(1) if an application was approved for the drug
13 under section 505(b), a certificate was issued for the
14 drug under section 507, or a license was issued for the
15 drug under section 351 of the Public Health Service
16 Act, the manufacturer of the drug will notify the Sec-
17 retary of any discontinuance of the production of the
18 drug at least one year before discontinuance, and

19 “(2) if an application has not been approved for
20 the drug under section 505(b), a certificate has not
21 been issued for the drug under section 507, or a license
22 has not been issued for the drug under section 351 of
23 the Public Health Service Act and if preclinical inves-
24 tigations or investigations under section 505(i) are
25 being conducted with the drug, the manufacturer or

1 *sponsor of the drug will notify the Secretary of any de-*
 2 *cision to discontinue active pursuit of approval of an*
 3 *application under section 505(b), approval of an appli-*
 4 *cation for certification under section 507, or approval*
 5 *of a license under section 351 of the Public Health*
 6 *Service Act.”.*

7 **SEC. 4. FINANCIAL ASSISTANCE.**

8 (a) **MEDICAL DEVICES.**—Section 5 of the Orphan
 9 Drug Act (21 U.S.C. 360ee) is amended—

10 (1) in subsection (a), by inserting “(1)” after
 11 “assist in” and by inserting before the period a comma
 12 and “(2) defraying the costs of developing medical de-
 13 vices for rare diseases or conditions”, and

14 (2) in subsection (b)(2)—

15 (A) by inserting “(A) in the case of a drug,”
 16 after “means” in the first sentence and by adding
 17 before the period in that sentence a comma and
 18 “(B) in the case of a medical device, any disease
 19 or condition that occurs so infrequently in the
 20 United States that there is no reasonable expecta-
 21 tion that a medical device for such disease or con-
 22 dition will be developed without assistance under
 23 subsection (a)”, and

24 (B) by striking out “under this subsection”
 25 in the last sentence and inserting in lieu thereof

1 *“under section 526 of the Federal Food, Drug,*
2 *and Cosmetic Act”.*

3 **(b) MEDICAL FOODS.**—*Section 5 of the Orphan Drug*
4 *Act (21 U.S.C. 360ee) is amended—*

5 *(1) in subsection (a) (as amended by subsection*
6 *(a)), by inserting before the period a comma and “and*
7 *(3) defraying the costs of developing medical foods for*
8 *rare diseases or conditions”,*

9 *(2) in subsection (b)(2) (as amended by subsec-*
10 *tion (a)), by inserting before the period at the end of*
11 *the first sentence a comma and “and (C) in the case of*
12 *a medical food, any disease or condition that occurs so*
13 *infrequently in the United States that there is no rea-*
14 *sonable expectation that a medical food for such disease*
15 *or condition will be developed without assistance under*
16 *subsection (a)”, and*

17 *(3) by adding at the end of subsection (b) the*
18 *following:*

19 *“(3) The term ‘medical food’ means a food which*
20 *is formulated to be consumed or administered enterally*
21 *under the supervision of a physician and which is in-*
22 *tended for the specific dietary management of a disease*
23 *or condition for which distinctive nutritional require-*
24 *ments, based on recognized scientific principles, are es-*
25 *tablished by medical evaluation.”.*

1 (c) *QUALIFICATION FOR ASSISTANCE.*—Section 5(b)
2 of the Orphan Drug Act (21 U.S.C. 360ee(b)) is amended—

3 (1) by striking out “and before the date on which
4 an application with respect to such drug is submitted
5 under section 505(b) or 507 of such Act or under sec-
6 tion 351 of the Public Health Service Act” in para-
7 graphs (1)(A)(ii) and (1)(B), and

8 (2) by striking out “and” at the end of subpara-
9 graph (A), by striking out the period at the end of sub-
10 paragraph (B) and inserting in lieu thereof “; and”,
11 and by adding after subparagraph (B) the following:

12 “(C) in the case of a drug which has not
13 been designated under section 526 of the Federal
14 Food, Drug, and Cosmetic Act for a rare disease
15 or condition but which the Secretary has reason
16 to believe is a drug for a rare disease or condition,
17 human clinical testing described in subparagraph
18 (A)(i) and preclinical testing.”.

19 (d) *AUTHORIZATION.*—Section 5(c) of the Orphan
20 Drug Act (21 U.S.C. 360ee(c)) is amended to read as
21 follows:

22 “(c) For grants and contracts under subsection (a) there
23 are authorized to be appropriated \$10,000,000 for fiscal year
24 1988, \$12,000,000 for fiscal year 1989, \$14,000,000 for
25 fiscal year 1990.”.

1 (d) *STUDY.*—*The Secretary of Health and Human*
2 *Services shall conduct a study to determine whether the ap-*
3 *plication of subchapter B of chapter V of the Federal Food,*
4 *Drug, and Cosmetic Act (relating to drugs for rare diseases*
5 *and conditions) and section 28 of the Internal Revenue Code*
6 *of 1986 (relating to tax credit) to medical devices or medical*
7 *foods for rare diseases or conditions or to both is needed to*
8 *encourage the development of such devices and foods. The*
9 *Secretary shall report the results of the study to the Commit-*
10 *tee on Energy and Commerce of the House of Representa-*
11 *tives and the Committee on Labor and Human Resources of*
12 *the Senate not later than one year after the date of the enact-*
13 *ment of this Act. For purposes of this section, the term “rare*
14 *diseases or conditions” has the meaning prescribed by section*
15 *5 of the Orphan Drug Act (21 U.S.C. 360ee).*

16 **SEC. 5. NATIONAL COMMISSION ON ORPHAN DISEASES.**

17 Section 4(n) of the Orphan Drug Amendments of 1985
18 (42 U.S.C. 236 note) is amended by striking out “Sep-
19 tember 30, 1987” and inserting in lieu thereof “February
20 1, 1989”.

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